



The undersigned requests that the present international application be processed according to the Patent Cooperation Treaty.

For receiving Office use only

International Application No.

International Filing Date

04 JAN 2005

Name of receiving Office and "PCT International Application"

Applicant's or agent's file reference
(if desired) (12 characters maximum) PC445PR

Box No. I TITLE OF INVENTION

"A novel use of rapamycin and structural analogues thereof"

Box No. II APPLICANT

☐ This person is also inventor

Name and address: (Family name followed by given name; for a legal entity, full official designation. The address must include postal code and name of country. The country of the address indicated in this Box is the applicant's State (that is, country) of residence if no State of residence is indicated below.)

UNIVERSITA' DEGLI STUDI DI FERRARA
Via Savonarola 9,
I-44100 FERRARA (Italy)

Telephone No.

Facsimile No.

Teleprinter No.

Applicant's registration No. with the Office

State (that is, country) of nationality:
IT

State (that is, country) of residence:
IT

This person is applicant for the purposes of: ☐ all designated States ☒ all designated States except the United States of America ☐ the United States of America only ☐ the States indicated in the Supplemental Box

Box No. III FURTHER APPLICANT(S) AND/OR (FURTHER) INVENTOR(S)

Name and address: (Family name followed by given name; for a legal entity, full official designation. The address must include postal code and name of country. The country of the address indicated in this Box is the applicant's State (that is, country) of residence if no State of residence is indicated below.)

ASSOCIAZIONE VENETA PER LA LOTTA ALLA
TALASSEMIA
Centro Microcitemia Dell'Azienda ULSS 18
I-45100 ROVIGO (Italy)

This person is:

☒ applicant only

☐ applicant and inventor

☐ inventor only (If this check-box is marked, do not fill in below.)

Applicant's registration No. with the Office

State (that is, country) of nationality:
IT

State (that is, country) of residence:
IT

This person is applicant for the purposes of: ☐ all designated States ☒ all designated States except the United States of America ☐ the United States of America only ☐ the States indicated in the Supplemental Box

☒ Further applicants and/or (further) inventors are indicated on a continuation sheet.

Box No. IV AGENT OR COMMON REPRESENTATIVE; OR ADDRESS FOR CORRESPONDENCE

The person identified below is hereby/has been appointed to act on behalf of the applicant(s) before the competent International Authorities as:

☒ agent

☐ common representative

Name and address: (Family name followed by given name; for a legal entity, full official designation. The address must include postal code and name of country.)

RAMBELLI, Paolo; JACOBACCI, Guido; QUINTERNO,
Giuseppe; GERBINO, Angelo; SERRA, Francesco;
FIORAVANTI, Corrado; DEAMBROGI, Edgardo;
JACOBACCI & PARTNERS SpA, Corso Regio Parco 27,
I-10152 TORINO (IT)

Telephone No.

(39) (011) 2440311

Facsimile No.

(39) (011) 2440311

Teleprinter No.

Agent's registration No. with the Office

☐ Address for correspondence: Mark this check-box where no agent or common representative is/has been appointed and the space above is used instead to indicate a special address to which correspondence should be sent.

| Continuation of Box 1 FURTHER APPLICANT(S) AND/OR (FURTHER) INVENTOR(S) | |
|---|---|
| <i>If none of the following sub-boxes is used, this sheet should not be included in the request.</i> | |
| Name and address: (Family name followed by given name; for a legal entity, full official designation. The address must include postal code and name of country. The country of the address indicated in this Box is the applicant's State (that is, country) of residence if no State of residence is indicated below.) BIANCHI, Nicoletta Via G. Matteotti 10/A, I-44020 MEZZOGORO (Ferrara) Italy | This person is: <input type="checkbox"/> applicant only <input checked="" type="checkbox"/> applicant and inventor <input type="checkbox"/> inventor only (If this check-box is marked, do not fill in below.) Applicant's registration No. with the Office |
| State (that is, country) of nationality: IT | State (that is, country) of residence: IT |
| This person is applicant for the purposes of: <input type="checkbox"/> all designated States <input type="checkbox"/> all designated States except the United States of America <input checked="" type="checkbox"/> the United States of America only <input type="checkbox"/> the States indicated in the Supplemental Box | |
| Name and address: (Family name followed by given name; for a legal entity, full official designation. The address must include postal code and name of country. The country of the address indicated in this Box is the applicant's State (that is, country) of residence if no State of residence is indicated below.) BORGATTI, Monica Via Menotti Cova 6 I-44100 FERRARA (Italy) | This person is: <input type="checkbox"/> applicant only <input checked="" type="checkbox"/> applicant and inventor <input type="checkbox"/> inventor only (If this check-box is marked, do not fill in below.) Applicant's registration No. with the Office |
| State (that is, country) of nationality: IT | State (that is, country) of residence: IT |
| This person is applicant for the purposes of: <input type="checkbox"/> all designated States <input type="checkbox"/> all designated States except the United States of America <input checked="" type="checkbox"/> the United States of America only <input type="checkbox"/> the States indicated in the Supplemental Box | |
| Name and address: (Family name followed by given name; for a legal entity, full official designation. The address must include postal code and name of country. The country of the address indicated in this Box is the applicant's State (that is, country) of residence if no State of residence is indicated below.) GAMBARI, Roberto Via Normandia 17 I-40132 BOLOGNA (Italy) | This person is: <input type="checkbox"/> applicant only <input checked="" type="checkbox"/> applicant and inventor <input type="checkbox"/> inventor only (If this check-box is marked, do not fill in below.) Applicant's registration No. with the Office |
| State (that is, country) of nationality: IT | State (that is, country) of residence: IT |
| This person is applicant for the purposes of: <input type="checkbox"/> all designated States <input type="checkbox"/> all designated States except the United States of America <input checked="" type="checkbox"/> the United States of America only <input type="checkbox"/> the States indicated in the Supplemental Box | |
| Name and address: (Family name followed by given name; for a legal entity, full official designation. The address must include postal code and name of country. The country of the address indicated in this Box is the applicant's State (that is, country) of residence if no State of residence is indicated below.) MISCHIATI, Carlo Via Don Minzoni 99 I-45030 ROVIGO (Italy) | This person is: <input type="checkbox"/> applicant only <input checked="" type="checkbox"/> applicant and inventor <input type="checkbox"/> inventor only (If this check-box is marked, do not fill in below.) Applicant's registration No. with the Office |
| State (that is, country) of nationality: IT | State (that is, country) of residence: IT |
| This person is applicant for the purposes of: <input type="checkbox"/> all designated States <input type="checkbox"/> all designated States except the United States of America <input checked="" type="checkbox"/> the United States of America only <input type="checkbox"/> the States indicated in the Supplemental Box | |
| <input type="checkbox"/> Further applicants and/or (further) inventors are indicated on another continuation sheet. | |

Box No. V DESIGNATION OF STATES

Mark the applicable check-boxes below; at least one must be marked

The following designations are hereby made under Rule 4.9(a):

Regional Patent

- ☒ **AP** ARIPO Patent: GH Ghana, GM Gambia, KE Kenya, LS Lesotho, MW Malawi, MZ Mozambique, SD Sudan, SL Sierra Leone, SZ Swaziland, TZ United Republic of Tanzania, UG Uganda, ZM Zambia, ZW Zimbabwe, and any other State which is a Contracting State of the Harare Protocol and of the PCT (if other kind of protection or treatment desired, specify on dotted line)
- ☒ **EA** Eurasian Patent: AM Armenia, AZ Azerbaijan, BY Belarus, KG Kyrgyzstan, KZ Kazakhstan, MD Republic of Moldova, RU Russian Federation, TJ Tajikistan, TM Turkmenistan, and any other State which is a Contracting State of the Eurasian Patent Convention and of the PCT
- ☒ **EP** European Patent: AT Austria, BE Belgium, BG Bulgaria, CH & LI Switzerland and Liechtenstein, CY Cyprus, CZ Czech Republic, DE Germany, DK Denmark, EE Estonia, ES Spain, FI Finland, FR France, GB United Kingdom, GR Greece, HU Hungary, IE Ireland, IT Italy, LU Luxembourg, MC Monaco, NL Netherlands, PT Portugal, RO Romania, SE Sweden, SI Slovenia, SK Slovakia, TR Turkey, and any other State which is a Contracting State of the European Patent Convention and of the PCT
- ☒ **OA** OAPI Patent: BF Burkina Faso, BJ Benin, CF Central African Republic, CG Congo, CI Côte d'Ivoire, CM Cameroon, GA Gabon, GN Guinea, GQ Equatorial Guinea, GW Guinea-Bissau, ML Mali, MR Mauritania, NE Niger, SN Senegal, TD Chad, TG Togo, and any other State which is a member State of OAPI and a Contracting State of the PCT (if other kind of protection or treatment desired, specify on dotted line)

National Patent (if other kind of protection or treatment desired, specify on dotted line):

- | | | |
|---|--|---|
| <input checked="" type="checkbox"/> AE United Arab Emirates | <input checked="" type="checkbox"/> HR Croatia | <input checked="" type="checkbox"/> OM Oman |
| <input checked="" type="checkbox"/> AG Antigua and Barbuda | <input checked="" type="checkbox"/> HU Hungary | <input checked="" type="checkbox"/> PG Papua New Guinea |
| <input checked="" type="checkbox"/> AL Albania | <input checked="" type="checkbox"/> ID Indonesia | <input checked="" type="checkbox"/> PH Philippines |
| <input checked="" type="checkbox"/> AM Armenia | <input checked="" type="checkbox"/> IL Israel | <input checked="" type="checkbox"/> PL Poland |
| <input checked="" type="checkbox"/> AT Austria | <input checked="" type="checkbox"/> IN India | <input checked="" type="checkbox"/> PT Portugal |
| <input checked="" type="checkbox"/> AU Australia | <input checked="" type="checkbox"/> IS Iceland | <input checked="" type="checkbox"/> RO Romania |
| <input checked="" type="checkbox"/> AZ Azerbaijan | <input checked="" type="checkbox"/> JP Japan | <input checked="" type="checkbox"/> RU Russian Federation |
| <input checked="" type="checkbox"/> BA Bosnia and Herzegovina | <input checked="" type="checkbox"/> KE Kenya | <input checked="" type="checkbox"/> SC Seychelles |
| <input checked="" type="checkbox"/> BB Barbados | <input checked="" type="checkbox"/> KG Kyrgyzstan | <input checked="" type="checkbox"/> SD Sudan |
| <input checked="" type="checkbox"/> BG Bulgaria | <input checked="" type="checkbox"/> KP Democratic People's Republic of Korea | <input checked="" type="checkbox"/> SE Sweden |
| <input checked="" type="checkbox"/> BR Brazil | <input checked="" type="checkbox"/> KR Republic of Korea | <input checked="" type="checkbox"/> SG Singapore |
| <input checked="" type="checkbox"/> BY Belarus | <input checked="" type="checkbox"/> KZ Kazakhstan | <input checked="" type="checkbox"/> SK Slovakia |
| <input checked="" type="checkbox"/> BZ Belize | <input checked="" type="checkbox"/> LC Saint Lucia | <input checked="" type="checkbox"/> SL Sierra Leone |
| <input checked="" type="checkbox"/> CA Canada | <input checked="" type="checkbox"/> LK Sri Lanka | <input checked="" type="checkbox"/> SY Syrian Arab Republic |
| <input checked="" type="checkbox"/> CH & LI Switzerland and Liechtenstein | <input checked="" type="checkbox"/> LR Liberia | <input checked="" type="checkbox"/> TJ Tajikistan |
| <input checked="" type="checkbox"/> CN China | <input checked="" type="checkbox"/> LS Lesotho | <input checked="" type="checkbox"/> TM Turkmenistan |
| <input checked="" type="checkbox"/> CO Colombia | <input checked="" type="checkbox"/> LT Lithuania | <input checked="" type="checkbox"/> TN Tunisia |
| <input checked="" type="checkbox"/> CR Costa Rica | <input checked="" type="checkbox"/> LU Luxembourg | <input checked="" type="checkbox"/> TR Turkey |
| <input checked="" type="checkbox"/> CU Cuba | <input checked="" type="checkbox"/> LV Latvia | <input checked="" type="checkbox"/> TT Trinidad and Tobago |
| <input checked="" type="checkbox"/> CZ Czech Republic | <input checked="" type="checkbox"/> MA Morocco | <input checked="" type="checkbox"/> TZ United Republic of Tanzania |
| <input checked="" type="checkbox"/> DE Germany | <input checked="" type="checkbox"/> MD Republic of Moldova | <input checked="" type="checkbox"/> UA Ukraine |
| <input checked="" type="checkbox"/> DK Denmark | <input checked="" type="checkbox"/> MG Madagascar | <input checked="" type="checkbox"/> UG Uganda |
| <input checked="" type="checkbox"/> DM Dominica | <input checked="" type="checkbox"/> MK The former Yugoslav Republic of Macedonia | <input checked="" type="checkbox"/> US United States of America |
| <input checked="" type="checkbox"/> DZ Algeria | <input checked="" type="checkbox"/> MN Mongolia | <input checked="" type="checkbox"/> UZ Uzbekistan |
| <input checked="" type="checkbox"/> EC Ecuador | <input checked="" type="checkbox"/> MW Malawi | <input checked="" type="checkbox"/> VC Saint Vincent and the Grenadines |
| <input checked="" type="checkbox"/> EE Estonia | <input checked="" type="checkbox"/> MX Mexico | <input checked="" type="checkbox"/> VN Viet Nam |
| <input checked="" type="checkbox"/> ES Spain | <input checked="" type="checkbox"/> MZ Mozambique | <input checked="" type="checkbox"/> YU Serbia and Montenegro |
| <input checked="" type="checkbox"/> FI Finland | <input checked="" type="checkbox"/> NI Nicaragua | <input checked="" type="checkbox"/> ZA South Africa |
| <input checked="" type="checkbox"/> GB United Kingdom | <input checked="" type="checkbox"/> NO Norway | <input checked="" type="checkbox"/> ZM Zambia |
| <input checked="" type="checkbox"/> GD Grenada | <input checked="" type="checkbox"/> NZ New Zealand | <input checked="" type="checkbox"/> ZW Zimbabwe |
| <input checked="" type="checkbox"/> GE Georgia | | |
| <input checked="" type="checkbox"/> GH Ghana | | |
| <input checked="" type="checkbox"/> GM Gambia | | |

Check-boxes below reserved for designating States which have become party to the PCT after issuance of this sheet

☐ ☐ ☐

Precautionary Designation Statement: In addition to the designations made above, the applicant also makes under Rule 4.9(b) all other designations which would be permitted under the PCT except any designation(s) indicated in the Supplemental Box as being excluded from the scope of this statement. The applicant declares that those additional designations are subject to confirmation and that any designation which is not confirmed before the expiration of 15 months from the priority date is to be regarded as withdrawn by the applicant at the expiration of that time limit. (Confirmation (including fees) must reach the receiving Office within the 15-month time limit.)

Box No. VI PRIORITY CLAIM

The priority of the following earlier application(s) is hereby claimed:

| Filing date of earlier application (day/month/year) | Number of earlier application | Where earlier application is: | | |
|---|----------------------------------|--|---|--|
| | | national application: country or Member of WTO | regional application:* regional Office | international application: receiving Office |
| item (1) (04.07.2002) July 04, 2002 | TO2002A000582 | ITALY | | |
| item (2) | | | | |
| item (3) | | | | |
| item (4) | | | | |
| item (5) | | | | |

☐ Further priority claims are indicated in the Supplemental Box.

The receiving Office is requested to prepare and transmit to the International Bureau a certified copy of the earlier application(s) (only if the earlier application was filed with the Office which for the purposes of this international application is the receiving Office) identified above as:

☐ all items ☐ item (1) ☐ item (2) ☐ item (3) ☐ item (4) ☐ item (5) ☐ other, see Supplemental Box

* Where the earlier application is an ARIPO application, indicate at least one country party to the Paris Convention for the Protection of Industrial Property or one Member of the World Trade Organization for which that earlier application was filed (Rule 4.10(b)(ii)):

Box No. VII INTERNATIONAL SEARCHING AUTHORITY

Choice of International Searching Authority (ISA) (if two or more International Searching Authorities are competent to carry out the international search, indicate the Authority chosen; the two-letter code may be used):

ISA / EP

Request to use results of earlier search; reference to that search (if an earlier search has been carried out by or requested from the International Searching Authority):


Date (day/month/year) Number Country (or regional Office)

Box No. VIII DECLARATIONS

The following declarations are contained in Boxes Nos. VIII (i) to (v) (mark the applicable check-boxes below and indicate in the right column the number of each type of declaration):

Number of
declarations

- | | | | | |
|-------------------------------------|--------------------|--|---|----------------|
| <input type="checkbox"/> | Box No. VIII (i) | Declaration as to the identity of the inventor | : | |
| <input checked="" type="checkbox"/> | Box No. VIII (ii) | Declaration as to the applicant's entitlement, as at the international filing date, to apply for and be granted a patent | : | 1 (follows) |
| <input type="checkbox"/> | Box No. VIII (iii) | Declaration as to the applicant's entitlement, as at the international filing date, to claim the priority of the earlier application | : | |
| <input checked="" type="checkbox"/> | Box No. VIII (iv) | Declaration of inventorship (only for the purposes of the designation of the United States of America) | : | 1 (follows) |
| <input type="checkbox"/> | Box No. VIII (v) | Declaration as to non-prejudicial disclosures or exceptions to lack of novelty | : | |

| Box No. IX CHECK LANGUAGE OF FILING | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
|--|--|---|---|----|--------|---|----------|---|----------|--|----------------------------|----|-------------------|--|------------------------|--|---|--|---|---|---|--|--|--|--|--|--|--|---|--|---|--|--|--|--|--|--|--|--|--|--|---|--|--|--|---|--|---|--|---|--|
| <p>This international application contains:</p> <p>(a) in paper form, the following number of sheets:</p> <table style="width: 100%; border-collapse: collapse;"> <tr> <td style="padding: 2px;">request (including declaration sheets)</td> <td style="text-align: right; padding: 2px;">5</td> </tr> <tr> <td style="padding: 2px;">description (excluding sequence listings and/or tables related thereto)</td> <td style="text-align: right; padding: 2px;">10</td> </tr> <tr> <td style="padding: 2px;">claims</td> <td style="text-align: right; padding: 2px;">1</td> </tr> <tr> <td style="padding: 2px;">abstract</td> <td style="text-align: right; padding: 2px;">1</td> </tr> <tr> <td style="padding: 2px;">drawings</td> <td style="text-align: right; padding: 2px;"></td> </tr> <tr> <td style="padding: 2px;">Sub-total number of sheets</td> <td style="text-align: right; padding: 2px;">17</td> </tr> <tr> <td style="padding: 2px;">sequence listings</td> <td style="text-align: right; padding: 2px;"></td> </tr> <tr> <td style="padding: 2px;">tables related thereto</td> <td style="text-align: right; padding: 2px;"></td> </tr> </table> <p><i>(for both, actual number of sheets if filed in paper form, whether or not also filed in computer readable form; see (c) below)</i></p> <p>Total number of sheets : 17</p> <p>(b) <input type="checkbox"/> only in computer readable form (Section 801(a)(i))</p> <p style="padding-left: 20px;">(i) <input type="checkbox"/> sequence listings</p> <p style="padding-left: 20px;">(ii) <input type="checkbox"/> tables related thereto</p> <p>(c) <input type="checkbox"/> also in computer readable form (Section 801(a)(ii))</p> <p style="padding-left: 20px;">(i) <input type="checkbox"/> sequence listings</p> <p style="padding-left: 20px;">(ii) <input type="checkbox"/> tables related thereto</p> <p>Type and number of carriers (diskette, CD-ROM, CD-R or other) on which are contained the</p> <p style="padding-left: 20px;"><input type="checkbox"/> sequence listings:</p> <p style="padding-left: 20px;"><input type="checkbox"/> tables related thereto:</p> <p><i>(additional copies to be indicated under items 9(ii) and/or 10(ii), in right column)</i></p> | request (including declaration sheets) | 5 | description (excluding sequence listings and/or tables related thereto) | 10 | claims | 1 | abstract | 1 | drawings | | Sub-total number of sheets | 17 | sequence listings | | tables related thereto | | <p>This international application is accompanied by the following item(s) (mark the applicable check-boxes below and indicate in right column the number of each item):</p> <table style="width: 100%; border-collapse: collapse;"> <tr> <td style="padding: 2px;">1. <input checked="" type="checkbox"/> fee calculation sheet</td> <td style="text-align: right; padding: 2px;">1</td> </tr> <tr> <td style="padding: 2px;">2. <input checked="" type="checkbox"/> original separate power of attorney (follow)</td> <td style="text-align: right; padding: 2px;">2</td> </tr> <tr> <td style="padding: 2px;">3. <input type="checkbox"/> original general power of attorney</td> <td style="text-align: right; padding: 2px;"></td> </tr> <tr> <td style="padding: 2px;">4. <input type="checkbox"/> copy of general power of attorney; reference number, if any:</td> <td style="text-align: right; padding: 2px;"></td> </tr> <tr> <td style="padding: 2px;">5. <input type="checkbox"/> statement explaining lack of signature</td> <td style="text-align: right; padding: 2px;"></td> </tr> <tr> <td style="padding: 2px;">6. <input checked="" type="checkbox"/> priority document(s) identified in Box No. VI as item(s): (1) (follows)</td> <td style="text-align: right; padding: 2px;">1</td> </tr> <tr> <td style="padding: 2px;">7. <input checked="" type="checkbox"/> translation of international application into (language): English (follows)</td> <td style="text-align: right; padding: 2px;">1</td> </tr> <tr> <td style="padding: 2px;">8. <input type="checkbox"/> separate indications concerning deposited microorganism or other biological material</td> <td style="text-align: right; padding: 2px;"></td> </tr> <tr> <td style="padding: 2px;">9. <input type="checkbox"/> sequence listings in computer readable form (indicate type and number of carriers)</td> <td style="text-align: right; padding: 2px;"></td> </tr> <tr> <td style="padding: 2px;">(i) <input type="checkbox"/> copy submitted for the purposes of international search under Rule 13ter only (and not as part of the international application):</td> <td style="text-align: right; padding: 2px;"></td> </tr> <tr> <td style="padding: 2px;">(ii) <input type="checkbox"/> (only where check-box (b)(i) or (c)(i) is marked in left column) additional copies including, where applicable, the copy for the purposes of international search under Rule 13ter</td> <td style="text-align: right; padding: 2px;"></td> </tr> <tr> <td style="padding: 2px;">(iii) <input type="checkbox"/> together with relevant statement as to the identity of the copy or copies with the sequence listings mentioned in left column</td> <td style="text-align: right; padding: 2px;"></td> </tr> <tr> <td style="padding: 2px;">10. <input type="checkbox"/> tables in computer readable form related to sequence listings (indicate type and number of carriers)</td> <td style="text-align: right; padding: 2px;"></td> </tr> <tr> <td style="padding: 2px;">(i) <input type="checkbox"/> copy submitted for the purposes of international search under Section 802(b-quater) only (and not as part of the international application)</td> <td style="text-align: right; padding: 2px;"></td> </tr> <tr> <td style="padding: 2px;">(ii) <input type="checkbox"/> (only where check-box (b)(ii) or (c)(ii) is marked in left column) additional copies including, where applicable, the copy for the purposes of international search under Section 802(b-quater)</td> <td style="text-align: right; padding: 2px;"></td> </tr> <tr> <td style="padding: 2px;">(iii) <input type="checkbox"/> together with relevant statement as to the identity of the copy or copies with the tables mentioned in left column</td> <td style="text-align: right; padding: 2px;"></td> </tr> <tr> <td style="padding: 2px;">11. <input type="checkbox"/> other (specify):</td> <td style="text-align: right; padding: 2px;"></td> </tr> </table> | 1. <input checked="" type="checkbox"/> fee calculation sheet | 1 | 2. <input checked="" type="checkbox"/> original separate power of attorney (follow) | 2 | 3. <input type="checkbox"/> original general power of attorney | | 4. <input type="checkbox"/> copy of general power of attorney; reference number, if any: | | 5. <input type="checkbox"/> statement explaining lack of signature | | 6. <input checked="" type="checkbox"/> priority document(s) identified in Box No. VI as item(s): (1) (follows) | 1 | 7. <input checked="" type="checkbox"/> translation of international application into (language): English (follows) | 1 | 8. <input type="checkbox"/> separate indications concerning deposited microorganism or other biological material | | 9. <input type="checkbox"/> sequence listings in computer readable form (indicate type and number of carriers) | | (i) <input type="checkbox"/> copy submitted for the purposes of international search under Rule 13ter only (and not as part of the international application): | | (ii) <input type="checkbox"/> (only where check-box (b)(i) or (c)(i) is marked in left column) additional copies including, where applicable, the copy for the purposes of international search under Rule 13ter | | (iii) <input type="checkbox"/> together with relevant statement as to the identity of the copy or copies with the sequence listings mentioned in left column | | 10. <input type="checkbox"/> tables in computer readable form related to sequence listings (indicate type and number of carriers) | | (i) <input type="checkbox"/> copy submitted for the purposes of international search under Section 802(b-quater) only (and not as part of the international application) | | (ii) <input type="checkbox"/> (only where check-box (b)(ii) or (c)(ii) is marked in left column) additional copies including, where applicable, the copy for the purposes of international search under Section 802(b-quater) | | (iii) <input type="checkbox"/> together with relevant statement as to the identity of the copy or copies with the tables mentioned in left column | | 11. <input type="checkbox"/> other (specify): | |
| request (including declaration sheets) | 5 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| description (excluding sequence listings and/or tables related thereto) | 10 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| claims | 1 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| abstract | 1 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| drawings | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| Sub-total number of sheets | 17 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| sequence listings | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| tables related thereto | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| 1. <input checked="" type="checkbox"/> fee calculation sheet | 1 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| 2. <input checked="" type="checkbox"/> original separate power of attorney (follow) | 2 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| 3. <input type="checkbox"/> original general power of attorney | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| 4. <input type="checkbox"/> copy of general power of attorney; reference number, if any: | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| 5. <input type="checkbox"/> statement explaining lack of signature | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| 6. <input checked="" type="checkbox"/> priority document(s) identified in Box No. VI as item(s): (1) (follows) | 1 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| 7. <input checked="" type="checkbox"/> translation of international application into (language): English (follows) | 1 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| 8. <input type="checkbox"/> separate indications concerning deposited microorganism or other biological material | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| 9. <input type="checkbox"/> sequence listings in computer readable form (indicate type and number of carriers) | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| (i) <input type="checkbox"/> copy submitted for the purposes of international search under Rule 13ter only (and not as part of the international application): | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| (ii) <input type="checkbox"/> (only where check-box (b)(i) or (c)(i) is marked in left column) additional copies including, where applicable, the copy for the purposes of international search under Rule 13ter | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| (iii) <input type="checkbox"/> together with relevant statement as to the identity of the copy or copies with the sequence listings mentioned in left column | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| 10. <input type="checkbox"/> tables in computer readable form related to sequence listings (indicate type and number of carriers) | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| (i) <input type="checkbox"/> copy submitted for the purposes of international search under Section 802(b-quater) only (and not as part of the international application) | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| (ii) <input type="checkbox"/> (only where check-box (b)(ii) or (c)(ii) is marked in left column) additional copies including, where applicable, the copy for the purposes of international search under Section 802(b-quater) | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| (iii) <input type="checkbox"/> together with relevant statement as to the identity of the copy or copies with the tables mentioned in left column | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| 11. <input type="checkbox"/> other (specify): | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| Figure of the drawings which should accompany the abstract: none | Language of filing of the international application: Italian | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| Box No. X SIGNATURE OF APPLICANT, AGENT OR COMMON REPRESENTATIVE <i>Next to each signature, indicate the name of the person signing and the capacity in which the person signs (if such capacity is not obvious from reading the request).</i> | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
|  RAMBELLI, Paolo | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |

| For receiving Office use only | |
|---|--|
| 1. Date of actual receipt of the purported international application: | 2. Drawings: <input type="checkbox"/> received: <input type="checkbox"/> not received: |
| 3. Corrected date of actual receipt due to later but timely received papers or drawings completing the purported international application: | |
| 4. Date of timely receipt of the required corrections under PCT Article 11(2): | |
| 5. International Searching Authority (if two or more are competent): ISA / | 6. <input type="checkbox"/> Transmittal of search copy delayed until search fee is paid |

| For International Bureau use only |
|---|
| Date of receipt of the record copy by the International Bureau: |

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
15 January 2004 (15.01.2004)

PCT

(10) International Publication Number
WO 2004/004697 A3

(51) International Patent Classification⁷: A61K 31/453,
31/203, 31/7068, 31/704, 31/17, 31/708

(21) International Application Number:
PCT/IB2003/002632

(22) International Filing Date: 3 July 2003 (03.07.2003)

(25) Filing Language: Italian

(26) Publication Language: English

(30) Priority Data:
TO2002A000582 4 July 2002 (04.07.2002) IT

(71) Applicants (for all designated States except US): UNI-
VERSITA' DEGLI STUDI DI FERRARA [IT/IT]; Via
Savonarola, 9, I-44100 Ferrara (IT). ASSOCIAZIONE
VENETA PER LA LOTTA ALLA [IT/IT]: c/o Centro
Microcitemia Dell'Azienda ULSS 18, I-45100 Rovigo
(IT).

(72) Inventors; and

(75) Inventors/Applicants (for US only): BIANCHI, Nico-
letta [IT/IT]; Via G. Matteotti 10/A, I-44020 Mezzogoro
(IT). BORGATTI, Monica [IT/IT]; Via Menotti Cova
6, I-44100 Ferrara (IT). GAMBARI, Roberto [IT/IT];
Via Normandia, 17, I-40132 Bologna (IT). MISCHIATI,
Carlo [IT/IT]; Via Don Minzoni, 99, I-45030 Rovigo (IT).

(74) Agents: RAMBELLI, Paolo et al.; Jacobacci & Partners
SpA, Corso Regio Parco 27, I-10152 Torino (IT).

(81) Designated States (national): AE, AG, AL, AM, AT, AU,
AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU,
CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH,
GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC,
LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW,
MX, MZ, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC,
SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA,
UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.

(84) Designated States (regional): ARIPO patent (GH, GM,
KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW),
Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM),
European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE,
ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO,
SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM,
GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Declaration under Rule 4.17:

— of inventorship (Rule 4.17(iv)) for US only

Published:

— with international search report
— before the expiration of the time limit for amending the
claims and to be republished in the event of receipt of
amendments

(88) Date of publication of the international search report:
25 March 2004

For two-letter codes and other abbreviations, refer to the "Guid-
ance Notes on Codes and Abbreviations" appearing at the begin-
ning of each regular issue of the PCT Gazette.

WO 2004/004697 A3

(54) Title: A NOVEL USE OF RAPAMYCIN AND STRUCTURAL ANALOGUES THEREOF

(57) Abstract: The use of rapamycin and its structural analogues for the preparation of a medicament for the therapeutic treatment of beta-thalassaemia is described.

**This Page is Inserted by IFW Indexing and Scanning
Operations and is not part of the Official Record**

BEST AVAILABLE IMAGES

Defective images within this document are accurate representations of the original documents submitted by the applicant.

Defects in the images include but are not limited to the items checked:

- ☒ **BLACK BORDERS**
- ☐ **IMAGE CUT OFF AT TOP, BOTTOM OR SIDES**
- ☐ **FADED TEXT OR DRAWING**
- ☐ **BLURRED OR ILLEGIBLE TEXT OR DRAWING**
- ☐ **SKEWED/SLANTED IMAGES**
- ☐ **COLOR OR BLACK AND WHITE PHOTOGRAPHS**
- ☒ **GRAY SCALE DOCUMENTS**
- ☐ **LINES OR MARKS ON ORIGINAL DOCUMENT**
- ☒ **REFERENCE(S) OR EXHIBIT(S) SUBMITTED ARE POOR QUALITY**
- ☐ **OTHER:** _____

IMAGES ARE BEST AVAILABLE COPY.

As rescanning these documents will not correct the image problems checked, please do not report these problems to the IFW Image Problem Mailbox.